

(12) INTERNATIONAL APPLICATION PUBLISHED UNDER THE PATENT COOPERATION TREATY (PCT)

(19) World Intellectual Property
Organization
International Bureau



(43) International Publication Date
17 June 2004 (17.06.2004)

PCT

(10) International Publication Number
WO 2004/050859 A3

- (51) International Patent Classification⁷: C12N 15/85, A61K 48/00 (74) Agent: LAWRENCE, William, F.; Frommer Lawrence & Haug LLP, 745 Fifth Avenue, New York, NY 10151 (US).
- (21) International Application Number: PCT/US2003/038811 (81) Designated States (*national*): AE, AG, AL, AM, AT, AU, AZ, BA, BB, BG, BR, BW, BY, BZ, CA, CH, CN, CO, CR, CU, CZ, DE, DK, DM, DZ, EC, EE, EG, ES, FI, GB, GD, GE, GH, GM, HR, HU, ID, IL, IN, IS, JP, KE, KG, KP, KR, KZ, LC, LK, LR, LS, LT, LU, LV, MA, MD, MG, MK, MN, MW, MX, MZ, NI, NO, NZ, OM, PG, PH, PL, PT, RO, RU, SC, SD, SE, SG, SK, SL, SY, TJ, TM, TN, TR, TT, TZ, UA, UG, US, UZ, VC, VN, YU, ZA, ZM, ZW.
- (22) International Filing Date: 25 November 2003 (25.11.2003)
- (25) Filing Language: English
- (26) Publication Language: English (84) Designated States (*regional*): ARIPO patent (BW, GH, GM, KE, LS, MW, MZ, SD, SL, SZ, TZ, UG, ZM, ZW), Eurasian patent (AM, AZ, BY, KG, KZ, MD, RU, TJ, TM), European patent (AT, BE, BG, CH, CY, CZ, DE, DK, EE, ES, FI, FR, GB, GR, HU, IE, IT, LU, MC, NL, PT, RO, SE, SI, SK, TR), OAPI patent (BF, BJ, CF, CG, CI, CM, GA, GN, GQ, GW, ML, MR, NE, SN, TD, TG).
- (30) Priority Data: 60/429,631 27 November 2002 (27.11.2002) US
- (71) Applicant (*for all designated States except US*): REGENTS OF THE UNIVERSITY OF MINNESOTA [US/US]; 110 Washington Avenue South, Minneapolis, MN 55455 (US).
- (72) Inventors; and (75) Inventors/Applicants (*for US only*): VERFAILLIE, Catherine [US/US]; 585 Cretin Avenue South, St. Paul, MN 55116 (US). LAKSHMIPATHY, Uma [US/US]; Stem Cell Institute, University of Minnesota, 420 Delaware Street SE, Minneapolis, MN 55455 (US).
- Published:
— with international search report
- (88) Date of publication of the international search report: 12 August 2004
- For two-letter codes and other abbreviations, refer to the "Guidance Notes on Codes and Abbreviations" appearing at the beginning of each regular issue of the PCT Gazette.*

(54) Title: HOMOLOGOUS RECOMBINATION IN MULTIPOTENT ADULT PROGENITOR CELLS

(57) Abstract: The invention relates to methods of altering gene expression by homologous recombination in a multipotent adult progenitor cell (MAPC). In particular, methods of producing a recombinant MAPC, of correcting a genetic defect in a mammal, of providing a functional and/or therapeutic protein to a mammal, and of transforming a MAPC are provided. MAPCs containing an exogenous DNA as well as recombinant MAPCs and their differentiated progeny are also provided.

WO 2004/050859 A3

INTERNATIONAL SEARCH REPORT

International application No.

PCT/US03/38811

A. CLASSIFICATION OF SUBJECT MATTER

IPC(7) : C12N 15/85; A61K 48/00

US CL : 435/325; 514/44

According to International Patent Classification (IPC) or to both national classification and IPC

B. FIELDS SEARCHED

Minimum documentation searched (classification system followed by classification symbols)

U.S. : 435/325; 514/44

Documentation searched other than minimum documentation to the extent that such documents are included in the fields searched

Electronic data base consulted during the international search (name of data base and, where practicable, search terms used)
STN: EMBASE BIOSIS CAPLUS

C. DOCUMENTS CONSIDERED TO BE RELEVANT

Category *	Citation of document, with indication, where appropriate, of the relevant passages	Relevant to claim No.
A	Database EMBASE on STN, LAKSHMIPATHY, U et al. 'A Non-viral Method for Efficient Transfection of Embryonic and Adult Stem Cells.' Blood, 16 November 2003, Vol. 102, No. 11, pp. 492b, see Abstract.	1-20



Further documents are listed in the continuation of Box C.



See patent family annex.

* Special categories of cited documents:

"A" document defining the general state of the art which is not considered to be of particular relevance

"E" earlier application or patent published on or after the international filing date

"L" document which may throw doubts on priority claim(s) or which is cited to establish the publication date of another citation or other special reason (as specified)

"O" document referring to an oral disclosure, use, exhibition or other means

"P" document published prior to the international filing date but later than the priority date claimed

"T" later document published after the international filing date or priority date and not in conflict with the application but cited to understand the principle or theory underlying the invention

"X" document of particular relevance; the claimed invention cannot be considered novel or cannot be considered to involve an inventive step when the document is taken alone

"Y" document of particular relevance; the claimed invention cannot be considered to involve an inventive step when the document is combined with one or more other such documents, such combination being obvious to a person skilled in the art

"&" document member of the same patent family

Date of the actual completion of the international search

09 May 2004 (09.05.2004)

Date of mailing of the international search report

28 MAY 2004

Name and mailing address of the ISA/US

Mail Stop PCT, Attn: ISA/US
Commissioner for Patents
P.O. Box 1450
Alexandria, Virginia 22313-1450

Facsimile No. (703) 872-9306

Authorized officer

Celine Qian, Ph.D.

Telephone No. (571) 272-1600